**A current state analysis of patient expectations, experiences, and outcomes for gene therapies. Implications for health technology assessment and post-marketing surveillance.**

**Background**

Health Technology Assessment is the process of evaluating the risks and benefits of a health technology, including medicines, vaccines and devices, to inform access and funding decisions across a product’s lifecycle: registration to reimbursement to post-marketing surveillance (1). As end-users of health technology, patient’s expectations, experiences and outcomes are critical but often poorly integrated into HTA processes. This is especially true for innovative therapies such as gene technologies (2). Gene therapies potentially provide life-changing health outcomes for patients and consequently long-term benefits to society and health care systems (2). Internationally, gaps in HTA frameworks do not address the uniqueness of gene therapies which translates into delayed patient access, inequitable funding models and short-sighted safety monitoring programs (2).

**Objective**

To develop a framework to address gaps in patient-reported expectations, experiences, and outcomes with gene therapy to inform future HTA and post-marketing surveillance methods.

**Rationale**

Gene therapies provide life-changing health outcomes for patients and consequently long-term benefits to society and health care systems (2). Given their uniqueness, contemporary evidence on what patient-centred data should be captured to inform both HTA and long-term outcomes for gene therapies (post-marketing surveillance or PMS) are lacking. This evidence is critical to the evolution and strengthening of traditional HTA and PMS approaches to improve safe and equitable patient access.

**Issues to be addressed**

Traditional approaches to HTA and PMS do not address the uniqueness of gene therapies thereby limiting regulator’s and payer’s understanding of the true real-world benefit of these therapies and potentially impeding patient access to these life-changing therapies. This manuscript will present a framework for addressing gaps in evidence sourced from both the academic literature and stakeholder input.

**Content**

1. **Introduction and background**
   1. Traditional approaches to capturing patient expectations, experiences and outcomes including current place in HTA and PMS.
   2. Uniqueness of gene therapies and challenges with current approaches.
2. **Methods**
   1. Scoping review
   2. Stakeholder engagement
3. **Findings**
   1. Key gaps in knowledge/evidence
   2. Themes to build framework.
4. **Future perspectives**
   1. Summary of findings
   2. Recommendations for future research

**Composition of manuscript research and writing team**

|  |  |  |  |
| --- | --- | --- | --- |
| Name and Qualifications | Affiliation | Country | Expertise |
| Jodie Hillen BPharm, MClinEpi, PhD  (Group Lead) | University of South Australia and Evohealth consulting | Australia | Pharmacoepidemiology, literature reviews, patient engagement and HTA. |
| Lourens Bloem PharmD, PhD | Utrecht University | Netherlands | Pharmacoepidemiology, HTA and CGTs. |
| Kui Huang PhD | Pfizer. Inc | US | Observational studies including LTFU studies in Gene Therapy and HTA. |
| Jamie Geier, PhD | Novartis | US | Observational studies and HTA. |
| Others…… |  |  |  |
|  |  |  |  |
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**Budget**

1. The manuscript writing team would like to request funding for open access publication. Cost estimated at $USD 2000.
2. The manuscript team would like to request funding for stakeholder honorariums ($USD 50 to 100 per interview = 1000)
3. The manuscript team would like to request funding for a research assistant to assist with data analytics and synthesis ($USD 3500).

**Target Journals**

1. Journal of Precision Medicine 2. Value in Health 3. Health Expectations 4. HTA 5. BMJ Open 6. International Journal for Quality in Health Care.

**Proposed timeline**

|  |  |  |
| --- | --- | --- |
| Date | Duration | Activity |
| Jan 2024 |  | Finalise manuscript methodology and outline. Assign team tasks. |
| Feb -Mar 2024 |  | Scoping review of literature.   * Refine search terms and inclusion/exclusion criteria. * Conduct database searches. * Screen identified literature and finalise search result flowchart. * Synthesis findings for the manuscript. |
| Jan 2024 |  | Identify suitable stakeholders to interview (n=10). |
| Feb 2024 |  | Finalise interview guide. |
| March 2024 |  | Submit interview guide for Human Research Ethics Committee approval. |
| April 2024 |  | Conduct interviews. |
| April 2024 |  | Synthesise interview findings into relevant themes. |
| May 2024 |  | Internal approval processes for writing team???? |
| June 2024 |  | Integrate interview and literature review findings and finalise manuscript for submission to ISPE Public Policy Committee. |

**Short biographies of writing team and statements on conflict of interest.**

**References:**

1. Health Technology Assessment International (HTAi). Who we are. Alberta, Canada: HTAi,; 2022 [Available from: <https://htai.org/about/>.

2. Besley S, Hnederson N, Napier M, Cole A, Hampson G. Country Scorecards: Health Technology Assessment of Gene Therapies. OHE Consulting Report, London. Office of Health Economics.; 2023.